

# **Unapproved Drug Workshop Pediatric Studies**

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# Objectives

- **Describe legislation involving pediatric studies**
- **Describe voluntary study program**
- **Describe mandatory study requirements**



# Pediatric Legislation

- **Voluntary**
  - **Best Pharmaceuticals for Children Act**
    - Signed into law January 4, 2002
    - Renewed pediatric exclusivity incentive originally in FDAMA
- **Mandatory**
  - **Pediatric Research Equity Act**
    - Signed December 3, 2003
    - Restored some important aspects from the Pediatric Rule, enjoined in 2002

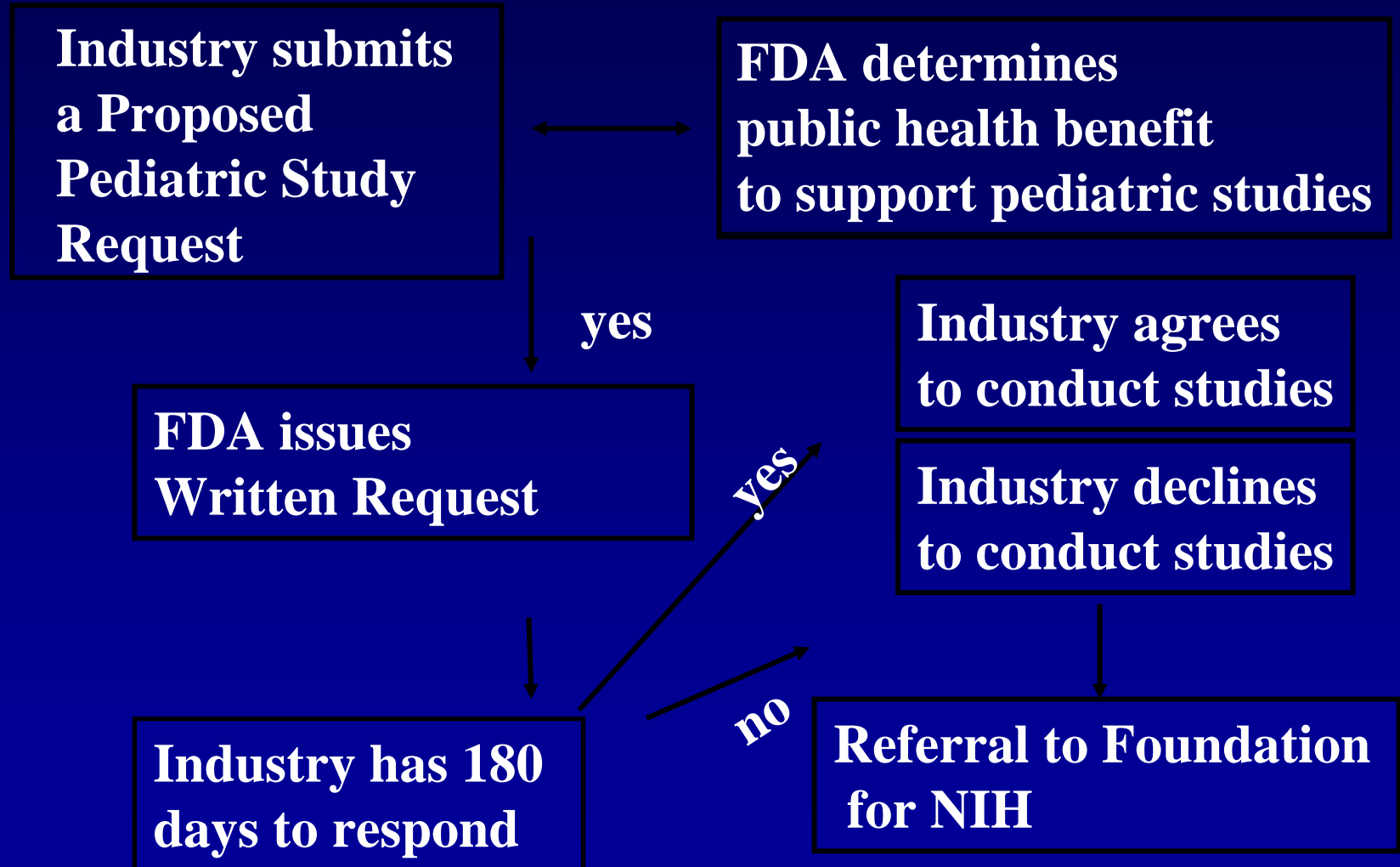


# **Best Pharmaceuticals for Children Act (BPCA)**

- **Sponsor submits a Proposed Pediatric Study Request (PPSR) outlining proposed study and public health benefit of conducting such study in pediatric patients**
- **FDA may issue a Written Request (WR) for Pediatric studies**
- **If studies are performed per the WR, 6 months of exclusivity will attach to the entire moiety**



# Process for the Study of On-Patent Drugs



# Pediatric Exclusivity

- 6 month period
- Attaches to existing patent or exclusivity
  - Not stand-alone exclusivity
- See “Qualifying for Pediatric Exclusivity Under Section 505A of the Federal Food, Drug, and Cosmetic Act “ Guidance for Industry
  - <http://www.fda.gov/cder/guidance/2891fnl.pdf>



# Pediatric Research Equity Act (PREA)

- **Assessment required for applications:**
  - New ingredient
  - New indication
  - New dosage form
  - New dosing regimen
  - New route of administration
- **Waiver or deferral may be granted**
- **Guidance for Industry “How to Comply with the Pediatric Research Equity Act”**
  - <http://www.fda.gov/cder/guidance/6215dft.pdf>



# Pediatric Assessment

**Assessment must contain:**

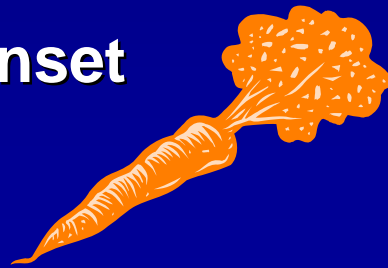
- **Data adequate to assess the safety and effectiveness of the drug or biological product, and**
- **Data to support dosing and administration for each subpopulation**



# BPCA vs. PREA

## BPCA

- Studies are voluntary
- Includes orphan drugs and orphan drug indications
- **Drugs only**
- Studies on whole moiety
- 10-1-07 Sunset



## PREA

- Studies are required
- Orphan drugs designated exempt
- **Biologics and Drugs**
- Studies limited to drug/indication under development
- 10-1-07 Sunset



# Conclusions

- **Two pieces of pediatric specific legislation**
- **Sponsors submitting applications need to be familiar with the requirements and incentives**
- **While they do not apply to all drugs, make sure obligations and opportunities have been discussed with review division**





# Back up Slides



# **PREA Waiver Requirements**

**Waiver granted when:**

- Necessary studies impossible or highly impracticable;**
- Strong evidence suggests the drug or biologic would be ineffective or unsafe; or**
- Product does not represent a meaningful therapeutic benefit over existing therapies and is not likely to be used in a substantial number of pediatric patients**



# **PREA Partial Waiver Requirements**

**Partial Waiver granted (applies to an age subset of the pediatric population) when:**

- Same criteria as waivers but with additional requirement**
- Reasonable attempts to produce a pediatric formulation necessary for that age group have failed**



# **PREA Deferral Requirements**

**Deferral granted when:**

- Drug or biologic is ready for approval in adults;**
- Additional safety and effectiveness data determined to be necessary; or**
- There is another appropriate reason for deferral**

